



AgiOS to Present Broad Set of Clinical and Translational Data for Oncology and Rare Genetic Disease Programs at the 2019 ASH Annual Meeting

November 6, 2019

– *Important Translational Data for TIBSOVO® Underscore the High Clinical and Molecular Remissions Rate Observed in Combination with Azacitidine in IDH1 Mutant AML and Expand Understanding of Resistance Mechanisms* –

– *Updated DRIVE PK Data for Mitapivat in PK Deficiency Patients Show Robust, Sustained Hemoglobin Responses in Extension Phase for More Than Three Years; New Data Characterize Comorbidities and Complications Associated with PK Deficiency* –

– *Company to Host Investor Event and Webcast on Monday, December 9, 2019 at 8:00p.m. ET* –

CAMBRIDGE, Mass., Nov. 06, 2019 (GLOBE NEWSWIRE) -- Agios Pharmaceuticals, Inc. (NASDAQ:AGIO), a leader in the field of cellular metabolism to treat cancer and rare genetic diseases, today announced that 15 abstracts featuring clinical and translational data from its oncology and rare genetic diseases programs will be presented at the American Society of Hematology (ASH) Annual Meeting being held December 7-10, 2019 in Orlando.

The accepted abstracts are listed below and are available online on the ASH conference website: <https://ash.confex.com/ash/2019/webprogram/start.html>.

Oral Presentations

Abstract #541: Complex Polyclonal Resistance Mechanisms to Ivosidenib Monotherapy in *IDH1*-Mutant Relapsed or Refractory Acute Myeloid Leukemia Revealed By Single Cell Sequencing Analyses

Date & Time: Monday, December 9, 2019 at 7:00 a.m. ET

Oral Abstract Session: 617. Acute Myeloid Leukemia: Biology, Cytogenetics, and Molecular Markers in Diagnosis and Prognosis: Single-Cell and Clonal Approaches to Treatment Resistance in AML

Location: Orange County Convention Center, Valencia A (W415A)

Presenter: Hongfang Wang, Ph.D., Agios Pharmaceuticals

Abstract #545: Molecular Mechanisms Mediating Relapse Following Ivosidenib Monotherapy in Patients with *IDH1*-Mutant Relapsed or Refractory Acute Myeloid Leukemia

Date & Time: Monday, December 9, 2019 at 8:00 a.m. ET

Oral Abstract Session: 617. Acute Myeloid Leukemia: Biology, Cytogenetics, and Molecular Markers in Diagnosis and Prognosis: Single-Cell and Clonal Approaches to Treatment Resistance in AML

Location: Orange County Convention Center, Valencia A (W415A)

Presenter: Sung Choe, Ph.D., Agios Pharmaceuticals

Abstract #643: Enasidenib Plus Azacitidine Significantly Improves Complete Remission and Overall Response Compared with Azacitidine Alone in Patients with Newly Diagnosed Acute Myeloid Leukemia (AML) with Isocitrate Dehydrogenase 2 (*IDH2*) Mutations: Interim Phase II Results from an Ongoing, Randomized Study

Date & Time: Monday, December 9, 2019 at 10:30 a.m. ET

Oral Abstract Session: 613. Acute Myeloid Leukemia: Clinical Studies: Non-Intensive Therapy

Location: Orange County Convention Center, Valencia A (W415A)

Presenter: Courtney DiNardo, M.D., University of Texas MD Anderson Cancer Center

Poster Presentations

Abstract #2175: Comorbidities and Complications in Adults with Pyruvate Kinase Deficiency

Poster Session Date & Time: Saturday, December 7, 2019 from 5:30-7:30 p.m. ET

Poster Session: 904. Outcomes Research – Non-Malignant Conditions: Poster II

Abstract #1570: Hematologic Malignancies Exhibit Selective Vulnerability to Inhibition of De Novo Pyrimidine Biosynthesis By AG-636, a Novel Inhibitor of Dihydroorotate Dehydrogenase in Phase 1 Clinical Trials

Poster Session Date & Time: Saturday, December 7, 2019 from 5:30-7:30 p.m. ET

Poster Session: 625. Lymphoma: Pre-Clinical – Chemotherapy and Biologic Agents: Poster I

Abstract #1286: AG-636 for the Treatment of Adults with Advanced Lymphoma: Initiation of a Phase 1 Clinical Study

Poster Session Date & Time: Saturday, December 7, 2019 from 5:30-7:30 p.m. ET

Poster Session: 605. Molecular Pharmacology, Drug Resistance – Lymphoid and Other Diseases: Poster I

Abstract #2223: An Ongoing Global, Longitudinal, Observational Study of Patients with Pyruvate Kinase Deficiency: The PEAK Registry

Poster Session Date & Time: Sunday, December 8, 2019 from 6:00-8:00 p.m. ET

Poster Session: 101. Red Cells and Erythropoiesis, Structure and Function, Metabolism, and Survival, Excluding Iron: Poster II

Abstract #2249: Mitapivat (AG-348), an Oral PK-R Activator, in Adults with Non-Transfusion Dependent Thalassemia: A Phase 2, Open-Label, Multicenter Study in Progress

Poster Session Date & Time: Sunday, December 8, 2019 from 6:00-8:00 p.m. ET

Poster Session: 112. Thalassemia and Globin Gene Regulation: Poster II

Abstract #3447: Development of Patient-Reported Outcome Measures (Symptoms and Impacts) in Adults with Pyruvate Kinase Deficiency

Poster Session Date & Time: Sunday, December 8, 2019 from 6:00-8:00 p.m. ET

Poster Session: 904. Outcomes Research – Non-Malignant Conditions: Poster II

Abstract #2593: AGILE: A Phase 3, Multicenter, Double-Blind, Randomized, Placebo-Controlled Study of Ivosidenib in Combination with Azacitidine in Adult Patients with Previously Untreated Acute Myeloid Leukemia with an IDH1 Mutation

Poster Session Date & Time: Sunday, December 8, 2019 from 6:00-8:00 p.m. ET

Poster Session: 613. Acute Myeloid Leukemia: Clinical Studies: Poster II

Abstract #2706: High Rate of *IDH1* Mutation Clearance and Measurable Residual Disease Negativity in Patients with *IDH1*-Mutant Newly Diagnosed Acute Myeloid Leukemia Treated with Ivosidenib (AG-120) and Azacitidine

Poster Session Date & Time: Sunday, December 8, 2019 from 6:00-8:00 p.m. ET

Poster Session: 617. Acute Myeloid Leukemia: Biology, Cytogenetics, and Molecular Markers in Diagnosis and Prognosis: Poster II

Abstract #3512: Long-Term Safety and Efficacy of Mitapivat (AG-348), a Pyruvate Kinase Activator, in Patients with Pyruvate Kinase Deficiency: The DRIVE PK Study

Poster Session Date & Time: Monday, December 9, 2019 from 6:00-8:00 p.m. ET

Poster Session: 101. Red Cells and Erythropoiesis, Structure and Function, Metabolism, and Survival, Excluding Iron: Poster III

Abstract #3526: Mitapivat (AG-348) in Adults with Pyruvate Kinase Deficiency Who Are Regularly Transfused: A Phase 3, Open-Label, Multicenter, Study (ACTIVATE-T) in Progress

Poster Session Date & Time: Monday, December 9, 2019 from 6:00-8:00 p.m. ET

Poster Session: 101. Red Cells and Erythropoiesis, Structure and Function, Metabolism, and Survival, Excluding Iron: Poster III

Abstract #3513: Prevalence of Pyruvate Kinase Deficiency: A Systematic Literature Review

Poster Session Date & Time: Monday, December 9, 2019 from 6:00-8:00 p.m. ET

Poster Session: 101. Red Cells and Erythropoiesis, Structure and Function, Metabolism, and Survival, Excluding Iron: Poster III

Abstract #4254: Ivosidenib (AG-120) in Patients with *IDH1*-Mutant Relapsed/Refractory Myelodysplastic Syndrome: Updated Enrollment of a Phase 1 Dose Escalation and Expansion Study

Poster Session Date & Time: Monday, December 9, 2019 from 6:00-8:00 p.m. ET

Poster Session: 637. Myelodysplastic Syndromes – Clinical Studies: Poster III

Event and Webcast Information

Agios will host an investor event on December 9, 2019 at 8:00 p.m. ET in Orlando to review the data from the company's oncology and rare genetic diseases programs. The event will be webcast live and can be accessed under "Events & Presentations" in the Investors section of the company's website at www.agios.com. The archived webcast will be available on the company's website beginning approximately two hours after the event.

About Agios

Agios is focused on discovering and developing novel investigational medicines to treat cancer and rare genetic diseases through scientific leadership in the field of cellular metabolism and adjacent areas of biology. In addition to an active research and discovery pipeline across both therapeutic areas, Agios has two approved oncology precision medicines and multiple first-in-class investigational therapies in clinical and/or preclinical development. All Agios programs focus on genetically identified patient populations, leveraging our knowledge of metabolism, biology and genomics. For more information, please visit the company's website at www.agios.com.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding the potential benefits of TIBSOVO® (ivosidenib) and mitapivat; Agios' plans regarding future data presentations; and the potential benefit of its strategic plans and focus. The words "anticipate," "expect," "intend," "potential," "milestone," "goal," "will," "on track," "upcoming," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from Agios' current expectations and beliefs. For example, there can be no guarantee that any product candidate Agios or its collaborators is developing will successfully commence or complete necessary preclinical and clinical development phases, or that development of any of Agios' product candidates will successfully continue. Moreover, there can be no guarantee that the two approved oncology precision medicines being commercialized by Agios and its collaborators will receive commercial acceptance. There can be no guarantee that any positive developments in Agios' business will result in stock price appreciation. Management's expectations and, therefore, any forward-looking statements in this press release could also be affected by risks and uncertainties relating to a number of other important factors, including: Agios' results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; the content and timing of decisions made by the U.S. FDA and other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies; Agios' ability to obtain and maintain requisite regulatory approvals and to enroll patients in its planned clinical trials; unplanned cash requirements and expenditures; competitive factors; Agios' ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates it is developing; Agios' ability to maintain key collaborations; and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in Agios' public filings with the Securities and Exchange Commission. Any forward-looking statements contained in this press release speak only as of the date hereof, and Agios expressly disclaims any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

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